Pharmacy Benefit Managers (PBMs)

Pharmacy Benefit Managers (PBMs) are third-party companies that act as intermediaries between insurance companies, drug manufacturers, and pharmacies. Their role is to manage prescription drug benefits on behalf of health insurers, large employers, and government programs. They determine what medications are covered, negotiate prices and rebates with drug manufacturers, and reimburse pharmacies for the drugs they dispense.

Why PBMs Are Problematic—Especially for the Rare Disease Community:

Rare disease advocacy groups are increasingly calling for **PBM reform** because of the outsized and often opaque influence PBMs have on drug access, affordability, and availability. Here's why:

1. **Lack of Transparency**
    - PBMs often negotiate large rebates with drug manufacturers but do **not pass those savings** directly to patients or even to the health plans.
    - These rebates are kept confidential, which makes it nearly impossible to understand how much a drug truly costs or where the money is going.
2. **Access Restrictions**
    - PBMs create **formularies** (lists of covered medications) that may **exclude or restrict access** to rare disease therapies, even when they are medically necessary.
    - Some rare disease drugs are placed on **higher tiers** with higher out-of-pocket costs, or require burdensome prior authorizations and step therapy ("fail first") protocols.
3. **Rebate-Driven Incentives**
    - PBMs may favor drugs that offer **higher rebates**, not necessarily the ones that are **most effective** or best suited for individual patients.
    - Rare disease treatments often **don't offer large rebates**, so they may be excluded or deprioritized on formularies.
4. **Financial Burden on Patients**
    - Patients with rare diseases can face **sky-high out-of-pocket costs**, even when a drug is technically covered, due to cost-sharing structures influenced by PBM decisions.
5. **Impact on Innovation**
    - PBM practices can discourage pharmaceutical companies from investing in rare disease treatments if they fear limited access or poor formulary placement, undermining innovation.

What PBM Reform Advocates Are Asking For:

Rare disease groups are pushing for reforms that include:

- Transparency in pricing and rebate practices
- Rebate pass-through to patients at the pharmacy counter
- Prohibition of "spread pricing" (where PBMs charge health plans more than they reimburse pharmacies and keep the difference)
- Limits on formulary exclusions and patient access restrictions for orphan and rare disease drugs
- More patient-centric formulary design

In short, PBM reform is critical to ensure that individuals with rare diseases can access the life-saving and life-changing therapies they need—without unnecessary delays, financial hardship, or denial of care due to profit-driven PBM practices.